EFGCP

EFGCP ANNUAL PAEDIATRIC CONFERENCE
BETTER MEDICINES FOR CHILDREN
COMMON ISSUES IN CLINICAL DEVELOPMENT AMPLIFIED IN PAEDIATRIC DRUG DEVELOPMENT

22 & 23 October 2024

Crowne Plaza Brussels Airport

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RATIONALE

Thank you for joining us!

The EFGCP "Better Medicines for Children" Conference is an annual flagship event providing a unique opportunity to share best practice, reflect on what has happened since we last met in October 2023, and discuss paediatric updates with a truly global outreach. Despite an ever changing and challenging environment, we believe we have been able to organise a dynamic, innovative, and highly engaging conference to inform our delegates of the latest scientific and regulatory developments in the paediatric space.

Last year, when we met in October 2023, gave us the opportunity to finally reconnect in person after 3 years of virtual conferences due to the pandemic. This year will also be an in-person conference to happen in Brussels, at the Crowne Plaza Airport Hotel.

The conference will focus on ***"Common issues in clinical development amplified in paediatric drug development"***, its challenges and solutions, and in particular its value, with a focus on what can be done and has been achieved in Europe, and this year also in the US and globally . To this effect, the conference will bring together distinguished speakers from all around the world and relevant stakeholders and experts involved in paediatric medicines development.

On **Day One**, further to a Key Note focusing on **Mental Health and Neurologic Disorders in Children**, there will be the opportunity to discuss implications of the new **Mechanism of Action Paediatric Investigation Plan**, which while considered a child-centric option, is a new option considered by Policy makers who are still working on the **revision of the Pharmaceutical Legislation**. **Examples of successful EU-funded paediatric projects.** One session will focus on multi-stakeholders' collaboration knowing a number of initiatives are already planned to start in 2024, which will benefit all involved in medicines development including children. The last session will provide the opportunity to discuss the **Potential of Artificial Intelligence in Paediatric Drug Development**

Active engagement of conference delegates will be welcomed in Breakout Sessions to share learnings, discuss challenges and identify solutions on what could be done to optimise childrens' access to new medicines, with a focus on the important topics:

1. Inclusion of Pregnant and Breastfeeding Individuals in Clinical Trials.
2. Use of Real-World Data/Registries in Paediatric Drug Development.
3. Paediatric-only medicines development/First in child companies.

Day Two will provide the opportunity to share **Paediatric Collaboration Activities at the Global Level** with a panel of regulators from various regions, such as Africa & Middle East, Japan, Australia, Canada, Europe, and the USA. This session will be complemented by a session on **Childrens' Access to Medicines in Developing Countries**. Prior to sharing the feedback from Day 1's Breakout Sessions, there will be an interesting session, **Health Technology Assessment (HTA) of Paediatric Medicines**, which will be of help to understand what sponsors will be facing with the implementation of the HTA Regulation in early 2025.

The Programme Committee looks forward to welcoming you all at the conference for a global educational and networking event that is a unique opportunity to learn from one another, to meet and interact with experienced colleagues and experts from all over of the world.

PROGRAMME COMMITTEE

Angeliki Siapkara	*AstraZeneca*	United Kingdom
Anja Schiel	*Norwegian Medicines Agency*	Norway
Begonya Nafria Escalera	*Barcelona Children's Hospital, eYPAGnet, EFGCP*	Spain
Chrissi Pallidis	*European Medicines Agency*	The Netherlands
Christina Kyriakopoulou	*European Commission*	Belgium
Claudio Fracasso	*Hippocrates Research Srl*	Italy
Gesine Bejeuhr	*Bayer*	Germany
Gilles Vassal	*Gustave Roussy Institute*	France
Kristina An Haack	*Sanofi*	France
Mark Turner	*Liverpool University Hospital*	United Kingdom
Martine Dehlinger-Kremer	*ICON, EUCROF, EFGCP*	Germany
Rhian Thomas-Turner	*Noah's Ark Children's Hospital for Wales*	United Kingdom
Sabine Fuerst-Recktenwald	*Roche*	Switzerland
Solange Corriol-Rohou	*AstraZeneca*	France
Thomas Severin	*Novartis*	Switzerland

FACULTY

Angelika Joos	*MDS*	Belgium
Argyris Stingaris	*University College of London*	United Kingdom
Benedetto Vitiello	*University of Turin*	Italy
Brian Aylward	*EMA, HPRA*	Ireland
Carmen Moreno	*Hospital Gregorio Maranon*	Spain
Cesare Spadoni	*Oncoheros*	Italy
Corinne de Vries	*EMA*	The Netherlands
Danielle Belgrave	*GSK*	United Kingdom
Dimitrios Athanasou	*EUPATI*	Greece
Emmanuella Amoako	*Yemaachi Biotech*	Ghana
Fabio D'Atri	*European Commission*	Belgium
Fahimeda Ali	*MHRA*	United kingdom
Jeffrey Barrett	*EMA*	The Netherlands
Katherine Donegan	*MHRA*	United kingdom
Lauren Wyatt	*ADHD Foundation*	United Kingdom
Lenias Hwenda	*Medicines for Africa Foundation*	South Africa
Louisa Braun Exner	*Danish Medicines Agency, PDCO Member*	Denmark
Lynne Yao	*FDA*	USA
Marc Ramis	*Venture Capital*	Spain
Marie Teil	*UCB Pharma*	Belgium
Marina Kolochavina	*Five Voices Consortium*	Germany
Martina Penazzato	*WHO*	Switzerland
Michiyo Sakiyama	*Pharmaceuticals and Medical Devices Agency*	Japan
Moy Bracken	*Access to Medicines Foundation*	The Netherlands
Natsuko Hayama	*Pharmaceuticals and Medical Devices Agency*	Japan
Roberto De Lisa	*EMA*	The Netherlands
Robyn Langham	*Therapeutic Goods Administration*	Australia
Sam Blackman	*Day One Biopharmaceutical*	USA
Sara Carucci	*Università degli studi di Cagliari*	Italy
Sylvie Benchetrit	*National Agency for the Safety of Medicine and Health Products*	France
Tyrza Boersma	*Patient representative*	The Netherlands
Ulrike Bonati	*Roche*	Switzerland
Victoria Kitcatt	*Pfizer*	United Kingdom
Yashwant Sinha	*Therapeutic Goods Administration*	Australia

ACKNOWLEDGMENTS

Thanks its Partners and Sponsors for their continued support!

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AGENDA

Day 1 - 22 October 2024

09:00 **REGISTRATION AND WELCOME COFFEE**

10:00 **Introduction to the Conference**

Martine Dehlinger-Kremer, *ICON & EUCROF*
Begonya Nafria-Escalera, *Barcelona Children's Hospital & eYPAGnet*

10:15 **KEY NOTE – Treatment of Psychiatric Disorders in children and adolescents: current situation and needs for research**

Carmen Moreno, *Hospital Gregorio Maranon*

10:45 **Session 1: Neuro Health**

Moderator: **Sabine Fuerst-Recktenwald,** *Roche* | **Sara Carucci,** *Università degli studi di Cagliari*

- Depression
  Argyris Stingaris, *University College of London* | **Louisa Braun Exner,** *PDCO representative Denmark*
- Multiple Sclerosis
  Ulrike Bonati, *Roche*
- Paediatric Irritability: From Definition and Assessment to Treatment
  Benedetto Vitiello, *University of Turin*

11:45 **Session 2: Mechanism of Action Paediatric Investigation Plan**

Moderators: **Gesine Bejeuhr,** *Bayer* and **Gilles Vassal,** *Gustave Roussy Institute*

Panelists: **Angelika Joos,** *MDS* | **Chrissi Pallidis,** *EMA* | **Sylvie Benchetrit,** *PDCO, EMA*

12:45 **LUNCH**

13:45 **Session 3: Examples of successful EU-funded paediatric projects**

Moderators: **Christina Kyriakopoulou,** *European Commission* and **Mark Turner,** *Liverpool University Hospital*

- VISION-DMD
- ALPHA-MAN
- Lena Project
- HIP PUMA

AGENDA

Day 1 - 22 October 2024

14:45 **Breakout sessions:**

1. Inclusion of Pregnant and Breastfeeding Individuals in Clinical Trials

Moderators: **Martine Dehlinger-Kremer,** *ICON & EUCROF* and **Solange Corriol-Rohou,** *AstraZeneca*

Panelists: **Corinne de Vries,** *EMA* | **Lynne Yao,** *FDA* | **Marie Teil,** *UCB* | **Tyrza Boersma,** *Patient representative*

2. Use of RWD/Registries in Paediatric Drug Development

Moderators: **Angeliki Siapkara,** *AstraZeneca* and **Jeffrey Barrett,** *Aridhia*

Panelists: **Katherine Donegan,** *MHRA* | **Marina Kolochavina,** *Five Voices Consortium* | **Roberto De Lisa,** *EMA*

3. Paediatric-only drug development First in child companies

Moderators: **Gesine Bejeuhr,** *Bayer* and **Rhian Thomas-Turner,** *Noah's Ark Children's Hospital forWales*

Panelists: **Cesare Sapon,** *Oncohereos* | **Kristina An Haack,** *Sanofi* | **Marc Ramis,** *Venture capital* | **Sam Blackman,** *Day One Biopharmaceutical*

16:30 **COFFEE BREAK**

17:00 **Session 4: The Potential of Artificial Intelligence in Paediatric Drug Development**

Moderator: **Claudio Fracasso,** *Hippocrates Research Srl* and **Thomas Severin,** *Novartis*

Panelists: **Danielle Belgrave,** *GSK* | **Kristina An Haack,** *Sanofi* | **Roberto De Lisa,** *EMA*

18:00 **Reflection from the PDCO Chair**

Brian Aylward, *Health Products Regulatory Authority*

18:30 **End of Day 1**

Martine Dehlinger-Kremer, *ICON & EUCROF*

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Day 2 - 23 October 2024

08:00 **WELCOME COFFEE**

08:30 **Welcome and Debrief from Day 1**

Begonya Nafria-Escalera, *Barcelona Children's Hospital & eYPAGnet*
Martine Dehlinger-Kremer, *ICON & EUCROF*

08:45 **Session 5: Regulatory updates**

Moderators: **Fahimeda Ali,** *MHRA* and **Sabine Fuerst- Recktenwald,** *Roche*

Panelists: **Michiyo Sakiyama and Natsuko Hayama,** *Pharmaceuticals and Medical Devices Agency* | **Robyn Langham and Yashwant Sinha,** *Therapeutic Goods Administration*

09:45 **Session 6: Pathways to Medicines in Developing Countries**

Moderators: **Martina Penazzato,** *WHO* and **Rhian Thomas-Turner,** *Noah's Ark Children's Hospital for Wales*

Panelists: **Emmanuella Amoako,** *Yemaachi Biotech* | **Lenias Hwenda,** *Medicines for Africa Foundation* | **Moy Bracken,** *Access to Medicines Foundation*

- Access to Childhood Cancer Medicines (WHO)
- Access to Medicines Foundation. They have a published methodology on assessing medicines specifically in developing countries

10:30 **COFFEE BREAK**

11:00 **Session 7: Multistakeholder Collaboration in 2024**

Moderator: **Solange Corriol-Rohou,** *AstraZeneca*

Panelists: **Christina Kyriakopoulou,** *European Commission* | **Dimitrios Athanasiou** *EUPATI* | **Mark Turner,** *Liverpool University Hospital*

12:00 **LUNCH**

13:00 **Session 8: European Legislations impact on paediatric medicines**

Moderators**: Angeliki Siapkara,** *AstraZeneca* and **Dimitrios Athanasiou,** *EUPATI*

Panelist: **Anja Schiel,** *Norwegian Medicines Agency* | **Brian Aylward,** *Health Products Regulatory Authority* | **Mark Turner,** *Liverpool University Hospital* | **Victoria Kitcatt,** *Pfizer*

14:00 **Feedback from the Breakout Sessions:**

1. Inclusion of Pregnant and Breastfeeding Individuals in Clinical Trials
2. Use of RWD/Registries in Paediatric Drug Development
3. Paediatric-only drug development First in child companies

15:00 **End of Day 2 – Conclusion & Farewell**

Begonya Nafria-Escalera, *Barcelona Children's Hospital & eYPAGnet*
Martine Dehlinger-Kremer, *ICON & EUCROF*